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OBJECTIVES: Adults with Philadelphia chromosome-positive (Ph+) acute lymphoblastic leukaemia (ALL) who develop resistance or intolerance to first- and second-generation tyrosine-kinase inhibitors (TKIs) may be eligible for potentially curative allogeneic hematopoietic stem cell transplantation (alloHSCT) if remission of the disease is achieved. The third-generation TKI ponatinib has been shown to be safe and efficacious in TKI resistant and intolerant patients, with 47% achieving a major cytogenetic response (MCyR). Accordingly, ponatinib followed by alloHSCT in those who achieve MCyR represents a potential therapeutic alternative to best supportive care (BSC) with standard chemotherapy only. **METHODS:** A Markov cohort model was constructed to assess the cost-effectiveness after dasatinib failure of ponatinib followed by alloHSCT in patients who achieve MCyR, versus BSC. Direct medical costs for ponatinib, BSC, alloHSCT, monitoring and follow-up, and adverse events were considered from the perspective of the UK National Health Service. Treatment outcomes were estimated from data for the Ph+ ALL patients in a phase 2 ponatinib trial (PACE), alloHSCT recipients in the LALA-94 trial, and a historical cohort receiving BSC. In the absence of valuations for Ph+ ALL health states, utilities for blast-phase chronic myeloid leukaemia were used. Outcomes were evaluated in terms of life-years (LY) and quality-adjusted life-years (QALYs), and cost-effectiveness in terms of life-years gained (LYG) and QALYs gained. **RESULTS:** Patients in the ponatinib plus alloHSCT arm had higher overall survival (4.14 versus 0.32 LY) and QALYs (2.57 versus 0.09) than BSC, at an increased cost (£88,553 versus £21,208). Incremental cost-effectiveness ratios were £17,700/LYG and £27,200/QALY gained relative to BSC. **CONCLUSIONS:** Given the assumptions and limitation of this analysis, our results suggest ponatinib may offer improved survival and health-related quality-of-life by enabling patients with Ph+ ALL who have failed dasatinib to achieve remission and benefit from alloHSCT, at a moderate increase in cost compared with BSC.

PSY54

COST-EFFECTIVENESS ANALYSIS OF ROMIPLOSTIM FOR THE TREATMENT OF ADULT CHRONIC IMMUNE THROMBOCYTOPENIC PURPURA (ITP) IN BRAZIL

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OBJECTIVES: To assess the cost-effectiveness of romiplostim as treatment for adult ITP splenectomized patients with refractory disease or non-splenectomized patients with surgery contra-indication, in comparison with the use of Intravenous Immunoglobulin (IVIg) rescue therapy only, from the Brazilian private health care perspective. **METHODS:** A cost per response model was developed. Overall response rates were derived from the romiplostim clinical trials (Kuter 2008) and were weighted by the proportion of patients splenectomized or not. The use of IVIg as rescue therapy in both arms was derived from Pullarkat, 2009. Treatment cost was calculated assuming drug wastage for an average patient weight of 72.4Kg and height of 170cm. Bleeding rates according to severity and site (gastrointestinal, intracranial and other) were estimated for patients without response considering data from Weitz, 2012. Resources and procedures used for the treatment of bleeding events were based on a medical specialist group recommendation and costs were obtained from official Brazilian databases. Gynecological bleed was used as a proxy for costs of other inpatient bleeds. The analysis assumed 50% splenectomized patients and was performed over a time horizon of 24 weeks. **RESULTS:** The average cost per patient over 24 weeks was R$60,509 for romiplostim (including rescue therapy and bleeding events costs) and R$173,319 for IVIg. 83.2% and 7.1% of patients in the romiplostim and IVIg arm respectively achieved overall response, leading to a cost per response equal to R$72,727 for romiplostim compared with R$2,424,047 for IVIg as rescue therapy. **CONCLUSIONS:** The use of romiplostim in the treatment of ITP increases and maintains the platelet level of splenectomized and non-splenectomized patients and, at the same time, reduces the need of rescue therapy as IVIg. That generates cost savings and positions romiplostim as a dominant (less costly and more effective) strategy when compared with IVIg rescue therapy only.

PSY55

RE-EVALUATING THE COST-EFFECTIVENESS OF SCREENING FOR CONGENITAL ADRENAL HYPERPLASIA (CAH): THE SENSITIVITY TO CHOICE OF DISTRIBUTIONS IN PROBABILISTIC SENSITIVITY ANALYSES (PSAS)

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OBJECTIVES: In 2009 Yoo and Grosse (Y/G) compared screening/no screening for CAH in a CEA, including a PSA using triangular distributions with minimum (MIN), mode and maximum (MAX) values for parameters. These distributions have been criticized for use in PSAs (Briggs et al). This research reproduces the Y/G analysis and then conducts a PSA with more appropriate distributional assumptions to evaluate potential bias from triangular distributions. **METHODS:** We limited our reanalysis to a change in distributions to focus on the question of distributional sensitivity. For parameters in Y/G with symmetric triangular distributions (MIN and MAX values equidistant from their mean/mode), we used a normal distribution with the same mean and chose a Standard Error based on a 95% confidence interval defined by the MIN and MAX values of the triangular. For non-symmetric distributions, we fit a beta distribution to the triangular, keeping the means equal and ensuring that the tails of the distributions extended slightly beyond the MIN and MAX of the triangular. **RESULTS:** We reproduced the Y/G deterministic ICER ($292,841 vs. Y/G reported $292,000). We also reproduced the triangular distribution-based PSA ICER from Y/G (256,947 vs Y/G $255,700). In our revised PSA, our mean ICER was higher ($273,187), but the CEACs were nearly superimposable. For low Willingness to Pay (WTP) values, the probability of being cost-effective - P(CE) - was very similar between the two analyses. There was some divergence at higher WTPs. For a WTP of $270,000, the P(CE) for the no screen option was .482 with the TRI distributions and .535 with our revised distributions. **CONCLUSIONS:** In this case, despite the criticism of triangular distributions generally, CEA results were not appreciably different qualitatively or quantitatively with the change to more accepted distributional assumptions. For an intervention with an ICER closer to the CE threshold, the importance may be greater.

PSY56

COST-EFFECTIVENESS ANALYSIS OF PONATINIB IN THE TREATMENT OF CHRONIC-PHASE CHRONIC MYELOID LEUKEMIA (CP-CML) IN SWEDEN

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OBJECTIVES: In CP-CML, there are few therapeutic options for highly-resistant patients (e.g. third line [3L] or beyond), who have a poor prognosis. Current treatment options are tyrosine kinase inhibitors (TKIs) and allogeneic stem cell transplantation (allo-SCT) for suitable patients. Efficacy of ponatinib, designed to inhibit the kinase activity of native BCR-ABL and all mutant variants, including T315I, was demonstrated in patients with highly-resistant CML in the pivotal phase II Ponatinib Ph+ ALL and CML Evaluation(PACE) trial. In the absence of head-to-head trials, an economic model employing a Swedish public healthcare perspective was developed to assess the cost-effectiveness of ponatinib for 3L treatment of CP-CML compared with current treatment options in Sweden. **METHODS:** The cost-effectiveness model compares ponatinib, second-generation TKIs (dasatinib, nilotinib, bosutinib), and allo-SCT, with cost per life-years (LY) saved and cost per quality-adjusted life-years (QALYs) gained as outcome measures, and a lifetime time horizon. Resource use includes study drugs, monitoring and follow-up, adverse events and allo-SCT procedure. Costs, based on current tariffs in Sweden, and benefits (LY and QALYs) projected based on 12-month treatment response, were discounted at 3%/year. We performed sensitivity analyses (SA) to identify parameters that most strongly influenced results. Clinical validity was evaluated by comparing model-generated survival estimates with relevant clinical data. **RESULTS:** Over the patients' lifetime, ponatinib provides an increase in LY of almost 7 years and a gain of almost 4 QALYs compared with the next-best therapy, bosutinib. The incremental cost-effectiveness ratios range from SEK78,044/QALY gained vs. allo-SCT to SEK351,702/QALY gained vs. bosutinib. SA showed the model was robust to plausible changes in input parameters and had good face validity. **CONCLUSIONS:** This analysis suggests that treating 3L CP-CML with ponatinib provides substantial clinical benefit as compared with current alternatives at a reasonable cost, from the perspective of the Swedish public healthcare system.

PSY57

THE COST-EFFECTIVENESS OF BIOLOGICS FOR THE TREATMENT OF INFLAMMATORY BOWEL DISEASES: A SYSTEMATIC REVIEW

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OBJECTIVES: Biologics are used for the treatment of inflammatory bowel diseases (IBDs), Crohn´s disease (CD) and Ulcerative colitis (UC), while their costs are significant high. To allocate health spending efficiently, biologics for IBDs are an important target for cost-effectiveness analyses. The aim of this study was to systemically review the cost-effectiveness of biologics for IBDs and to evaluate the methodological quality of cost-effectiveness analyses. **METHODS:** The literature search was performed to Medline (Ovid), Cochrane Library and SCOPUS in June 2014. The cost-utility analyses of biologics for IBDs in adults were included in the review. Biologics were compared with conventional medical treatment, another biologic treatment, placebo and surgery. Data extraction form was designed beforehand. All costs were converted to 2014 euro. The methodological quality of the included studies was assessed by Drummond´s checklist, Philips´ checklist and Consolidated Health Economic Evaluation Reporting Standards (CHEERS) guideline. **RESULTS:** Of the 1828 references found in the literature search, 25 studies were included in the final review. The main causes affected the cost-effectiveness of the biologics are the activity of the disease, the duration of the biological treatment and the treatment strategy. Among patients refractory to conventional medical treatment (CMT) for IBD, incremental cost-effectiveness ratio (ICER) ranged from dominance to €549,335 per Quality-Adjusted Life Year (QALY) compared with CMT. Adalimumab produced more frequently lower ICERs than infliximab in comparison with CMT for CD. When compared biologics with another biologic treatment for CD, ICER ranged from dominance to €24,012,483/QALY. A study including both direct and indirect costs produced more favorable ICERs than studies including only direct costs. **CONCLUSIONS:** With a threshold of €35,000/QALY, current evidence showed that biologics are cost-effective for the induction treatment of active and severe IBD. Biologics were not cost-effective for moderate IBD. Between biologics the cost-effectiveness remains unclear.

PSY58

IS REGENERATIVE MEDICINE COST-EFFECTIVE? EVIDENCES FROM THE FIRST APPROVED STEM CELL BASED PRODUCT

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OBJECTIVES: A cost-effectiveness analysis (CEA) has been performed, from an Italian public payer perspective, to compare the first advanced therapy medicinal product (ATMP) containing stem cells for the treatment of Limbal Stem Cell Deficiency (LSCD) with conservative treatment, a standard alternative pathway. LSCD is a rare condition characterized by the shortage of limbal stem cells in the eye resulting in corneal conjunctivalization, corneal opacity, visual impairment and even blindness. A medicinal product has been recently approved by EMA as the first treatment in moderate-severe LSCD due to chemical or physical burn. **METHODS:** Efficacy data derive from a retrospective, case-series, non-randomized, non-con-